Scholar Rock Reports Second Quarter 2022 Financial Results and Highlights Business Progress

August 8, 2022

- Positive 24-month data from ongoing Phase 2 TOPAZ trial extension supports potential benefit of apitegromab for patients with Types 2 and 3 Spinal Muscular Atrophy (SMA)

- Actively enrolling in pivotal Phase 3 SAPPHIRE clinical trial for apitegromab

- Discovery pipeline advancements made for LTBP-TGFβ1 target to treat a wide range of fibrotic diseases

- Significantly strengthened balance sheet with the completion of $205 million registered direct offering

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 8, 2022-- Scholar Rock (NASDAQ: SRRK), a Phase 3 clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, today reported financial results and corporate updates for the second quarter ended June 30, 2022.

"We are pleased to have reported important progress with our two clinical programs during the second quarter. Enrollment continues to advance in our pivotal Phase 3 SAPPHIRE trial for apitegromab in SMA. We also presented new data from our Phase 2 TOPAZ trial highlighting sizeable and sustained improvement in Hammersmith Functional Motor Scale-Expanded (HFMSE) scores at 24 months for non-ambulatory patients, reinforcing our belief in the unique potential of apitegromab as a muscle-targeted therapy to treat SMA, and we continue to evaluate these patients in their third year of treatment," said Nagesh Mahanthappa, Founding Chief Executive Officer & President of Scholar Rock. "In our SRK-181 program, enrollment is ongoing in the Phase 1 DRAGON trial Part B dose expansion. We look forward to providing updates on DRAGON as data become available. Further, our focused research continues to make excellent progress as we develop next-generation programs to build out our future pipeline."

Company Highlights and Upcoming Milestones

Apitegromab *is a selective inhibitor of myostatin activation being developed as the first muscle-targeted therapy with the potential to treat spinal muscular atrophy (SMA).*

- **Positive 24-Month Phase 2 TOPAZ Extension Data Support Potential Benefit for Patients.** Scholar Rock announced positive data at the Cure SMA Research & Clinical Care Meeting in June, demonstrating sizable and sustained motor function improvement at 24 months with apitegromab as measured by Hammersmith Functional Motor Scale-Expanded (HFMSE) scores for non-ambulatory patients with Types 2 and 3 SMA on nusinersen. Results also showed a substantial increase in Revised Upper Limb Module (RULM) scores, with no serious safety risks identified over 24 months of apitegromab treatment. Of the 55 patients who completed the 24-month extension period, 54 opted to continue into the 36-month extension period.
- **Enrollment Ongoing for Phase 3 SAPPHIRE Clinical Trial Evaluating Apitegromab in Non-Ambulatory Patients with Types 2 and 3 SMA.** The randomized, double-blind, placebo-controlled Phase 3 clinical trial is evaluating apitegromab for patients on either nusinersen or risdiplam. Approximately 156 non-ambulatory patients aged 2-12 years old with Types 2 and 3 SMA are planned to be enrolled in the main efficacy population. Patients will be randomized 1:1:1 to receive for 12 months either apitegromab 20 mg/kg, apitegromab 10 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks. SAPPHIRE is expected to be conducted across 55 sites in the U.S. and Europe. Scholar Rock presented on the trial design at the 17th International Congress on Neuromuscular Diseases (ICNMD 2022) in July 2022, along with publication of the abstract in the peer-reviewed *Journal of Neuromuscular Diseases*.

SRK-181 *is a selective inhibitor of latent TGFβ1 activation being developed with the aim of overcoming primary resistance to and increasing the number of patients who may benefit from checkpoint inhibitor therapy.*

- **Advancing Enrollment for Part B of the Phase 1 DRAGON Proof-of-Concept Clinical Trial for SRK-181.** Part B of the Phase 1 DRAGON trial consists of multiple proof-of-concept cohorts focused on evaluating the ability of SRK-181 to overcome primary resistance to anti-PD-(L)1 therapy in patients with solid tumors. The biomarker strategy for DRAGON explores early signs of SRK-181 activity, including target engagement and pathway modulation. It includes measuring effects on both circulating and tumor immune contexture, such as CD8+ T cell infiltration and reductions in myeloid-derived suppressor cell (MDSC) populations, as well as analysis of TGFβ-related pathway signaling. Initial evidence of drug activity and safety data are anticipated in 2022.

Scholar Rock's preclinical discovery pipeline includes a highly selective, context-dependent TGFβ1 antibody that inhibits proTGFβ1 activation selectively in the extracellular matrix via targeting the covalent complexes of proTGFβ1 with latent TGFβ binding proteins 1 and 3 ("LTBPs"— the targets are collectively referred to as "LTBP-TGFβ1").

- **Preclinical Data Supports Selective Targeting of Matrix-Associated TGF*β*1 as an Attractive Approach for Anti-Fibrotic Therapy.** Scholar Rock recently presented data on its targeted approach to LTBP-TGFβ1 that show reduction of TGFβ1 signaling and reduction of fibrosis in relevant preclinical *in vivo* models.

Second Quarter 2022 Financial Results

For the quarter ended June 30, 2022, net loss was $44.0 million or $1.06 per share compared to a net loss of $30.7 million or $0.84 per share for the quarter ended June 30, 2021.

- Revenue was $0 for the quarter ended June 30, 2022, compared to $4.6 million for the quarter ended June 30, 2021.
- Research and development expense was $32.1 million for the quarter ended June 30, 2022, compared to $25.6 million for the quarter ended June 30, 2021. The increase was primarily attributable to increased clinical costs associated with apitegromab and higher personnel costs, including severance expenses associated with the recent restructuring.
- General and administrative expense was $11.1 million for the quarter ended June 30, 2022, compared to $9.3 million for the quarter ended June 30, 2021. The increase was primarily due to higher personnel costs, including severance expenses associated with the recent restructuring.
- As of June 30, 2022, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $371 million, which is expected to fund the Company's anticipated operating and capital expenditure requirements into 2025.

"We were thrilled to announce the completion of a $205 million registered direct offering during the quarter. This financing included several high-quality, long-term oriented, fundamentals-based biotechnology investors who saw the promise of our platform and our programs. Importantly, this capital puts us in a strong financial position to fully fund the Phase 3 SAPPHIRE trial, continue advancing Part B of the Phase 1 DRAGON trial for SRK-181, while investing in selected early-stage programs that exemplify the power of our scientific platform," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock.

About Scholar Rock

Scholar Rock is a clinical-stage biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Scholar Rock is creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, and fibrosis. Scholar Rock's approach to targeting the molecular mechanisms of growth factor activation enabled it to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target these signaling proteins at the cellular level. By developing product candidates that act in the disease microenvironment, the Company intends to avoid the historical challenges associated with inhibiting growth factors for therapeutic effect. Scholar Rock believes its focus on biologically validated growth factors may facilitate a more efficient development path. For more information, please visit www.ScholarRock.com or follow Scholar Rock on Twitter (@ScholarRock) and LinkedIn (https://www.linkedin.com/company/scholar-rock/). Investors and others should note that we communicate with our investors and the public using our company website www.scholarrock.com, including, but not limited to, company disclosures, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference call transcripts and webcast transcripts, as well as on Twitter and LinkedIn. The information that we post on our website or on Twitter or LinkedIn could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

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Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its growth, strategy, progress and timing of its clinical trials for apitegromab, SRK-181, and other product candidates and indication selection and development timing, its cash runway, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. The use of words such as "may," "might," "could," "will," "should," "expect," "plan," "anticipate," "believe," "estimate," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify such forward-looking statements. All such forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, without limitation, that preclinical and clinical data, including the results from the Phase 2 clinical trial of apitegromab, or Part A of the Phase 1 clinical trial of SRK-181, are not predictive of, may be inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidate, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA or Part B of the Phase 1 clinical trial of SRK-181, Scholar Rock's ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline, the data generated from Scholar Rock's nonclinical and preclinical studies and clinical trials, information provided or decisions made by regulatory authorities, competition from third parties that are developing products for similar uses, Scholar Rock's ability to obtain, maintain and protect its intellectual property, Scholar Rock's dependence on third parties for development and manufacture of product candidates including, without limitation, to supply any clinical trials, Scholar Rock's ability to manage expenses and to obtain additional funding when needed to support its business activities and establish and maintain strategic business alliances and new business initiatives, and the impacts of public health pandemics such as COVID-19 on business operations and expectations, as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, as well as discussions of potential risks, uncertainties, and other important factors in Scholar Rock's subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock's views only as of today and should not be relied upon as representing its views as of any subsequent date. All information in this press release is as of the date of the release, and Scholar Rock undertakes

no duty to update this information unless required by law.

Scholar Rock Holding Corporation
Condensed Consolidated Statements of Operations
(unaudited)
(in thousands, except share and per share data)

	Three Months Ended June 30		Six Months Ended June 30	
	2022	2021	2022	2021
Revenue	$ —	$ 4,595	$ 33,193	$ 9,303
Operating expenses				
Research and development	32,073	25,603	61,439	48,152
General and administrative	11,074	9,265	21,834	18,631
Total operating expenses	43,147	34,868	83,273	66,783
Loss from operations	(43,147)	(30,273)	(50,080)	(57,480)
Other income (expense), net	(853)	(434)	(1,870)	(898)
Net loss	$ (44,000)	$ (30,707)	$ (51,950)	$ (58,378)
Net loss per share, basic and diluted	$ (1.06)	$ (0.84)	$ (1.31)	$ (1.60)
Weighted average common shares outstanding, basic and diluted	41,622,392	36,582,708	39,550,991	36,482,132

Scholar Rock Holding Corporation
Condensed Consolidated Balance Sheets
(unaudited)
(in thousands)

	June 30, 2022	December 31, 2021
Assets		
Cash, cash equivalents and marketable securities	$ 370,688	$ 252,994
Other current assets	18,451	12,325
Total current assets	389,139	265,319
Other assets	34,989	39,126
Total assets	$ 424,128	$ 304,445
Liabilities and Stockholders' Equity		
Current liabilities	$ 40,162	$ 64,297
Long-term liabilities	54,459	68,074
Total liabilities	94,621	132,371
Total stockholders' equity	329,507	172,074
Total liabilities and stockholders' equity	$ 424,128	$ 304,445

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